State of California—Health and Human Services Agency
Department of Health Care Services

RICHARD FIGUEROA
ACTING DIRECTOR

GAVIN NEWSOM
GOVERNOR

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TO: All County California Children's Services Program Administrators, Medical Consultants, and Integrated Systems of Care Division Staff

SUBJECT: Deflazacort (Emflaza) – Authorization Criteria (Revised)

I. PURPOSE

The purpose of this Numbered Letter (N.L.) is to update California Children's Services (CCS) Program policy regarding the authorization of deflazacort (Emflaza) as a treatment for Duchenne muscular dystrophy (DMD). This update clarifies that authorization criteria are consistent with age, dosage, and indication approved by the Food and Drug Administration (FDA).

II. BACKGROUND

DMD is a genetic disorder causing progressive muscular deterioration and weakness. This deterioration is caused by the absence or deficiency of dystrophin protein, which maintains intact muscle cells. DMD primarily affects skeletal, diaphragm, and heart muscle. DMD occurs in about 1 in 3,600 male infants worldwide with estimated incidence of 1 in 5,000 male infants in the United States. Symptoms appear between three and five years of age and progressively worsen over time. Affected individuals gradually lose their ability to perform daily activities, are usually wheelchair-bound by adolescence, and become ventilator-dependent patients by their 20s or 30s.

Deflazacort is a glucocorticoid that first launched in 1985 in Europe and has been used as an anti-inflammatory, and immunosuppressant in countries outside of the United States since the mid-1990s. On February 9, 2017, deflazacort received approval by the FDA for the treatment of the signs and symptoms of DMD. Compared to placebo, corticosteroids, including prednisone and deflazacort, have been shown to improve strength and pulmonary function in DMD.[1] However, compared to prednisone, deflazacort may be associated with less weight gain over the first years of treatment, and greater risk of cataracts.[2,3] The American Academy of Neurology recommends prednisone as the first-line steroid treatment for DMD.[3]

III. POLICY

Effective the date of this letter, the CCS Program may authorize deflazacort as a treatment for DMD if all the following criteria are met:

A. The client has a diagnosis of DMD, confirmed by genetic testing.

B. The client's care is under the supervision and monitoring of a CCS-approved Special Care Center (SCC) neurologist or physiatrist.

C. Prednisone, the preferred steroid treatment for DMD, has been discontinued due to:

   1. Adverse side effects or,

   2. No change in trajectory of disease progression after six months of use at recommended dosage.

D. The request is for the FDA-approved age and indication, and does not exceed the FDA-approved dosage.

E. If a CCS client has never been placed on prednisone as a treatment for DMD, the CCS Program shall authorize deflazacort only if the prescribing physician provides documentation that prednisone is likely to be ineffective and why, or medically contraindicated as a treatment for the client's DMD. This is because Title 22 of the California Code of Regulations, section 51003(f), requires the CCS Program to authorize the lowest cost drug that meets the beneficiary's medical needs, and the American Academy of Neurology recommends prednisone as the first-line steroid treatment for DMD.

F. If the criteria described above are not met, but the requesting provider has clinical documentation and/or scientific evidence that may be relevant to the request, the provider may submit this additional documentation to the Integrated Systems of Care Division (ISCD) Medical Director or designee for consideration during the eligibility determination.

IV. POLICY IMPLEMENTATION

A. Deflazacort is not covered by a Service Code Grouping authorization and a separate authorization is needed.

B. For all Deflazacort authorizations, requesting providers must submit a CCS Program Service Authorization Request (SAR) as follows:

   1. For clients residing in an independent county, requests shall be submitted to the CCS county office for processing.

2. For clients residing in a dependent county, requests shall be submitted to the Special Populations Unit by email at CCS_Operations@dhcs.ca.gov or via secure RightFax number, (916) 440-5768.

3. For clients residing in a county covered by the Whole Child Model, requests shall be submitted to, and processed by, the managed care plan.

C. For initial authorizations, the following must be submitted in addition to the SAR:

1. A copy of the prescription from the CCS-paneled SCC neurologist or physiatrist.

2. Progress notes from the neurology or rehabilitation SCC documenting genetic testing confirming the diagnosis of DMD, client weight and disease status, and 6 minute walk test (6MWT) or other objective assessment of ambulation.

3. Documentation of prior prednisone use including the dosage and length of time used. The specific reason(s) for discontinuation of prednisone must be listed with documentation describing the side effects. If never placed on prednisone, documentation explaining why prednisone is not an option must be provided.

4. Initial authorization shall be for six months.

D. Reauthorization shall be for up to 12 months. Requesting providers must submit a CCS Program SAR with current medical documentation demonstrating positive response to the medication to their county CCS program office replacement for this office along with:

1. Documentation that the client's weight or body mass index is stable or less than would be expected with the recommended dose of prednisone.

2. Documentation of client's ambulatory status, such as 6MWT.

3. For clients previously on prednisone, documentation that prednisone-related adverse effects have resolved with deflazacort use, or that the adverse effects are less than expected with prednisone.

4. At least annually, provide documentation of ophthalmology screening (potential or medical follow-up) for cataracts.

5. A report by registered dietician within six months of request.

If you have any questions regarding this N.L., please contact the ISCD Medical Director or designee, via email at ISCD-MedicalPolicy@dhcs.ca.gov.

Sincerely,

ORIGINAL SIGNED BY

Roy Schutzengel
Medical Director
Integrated Systems of Care Division

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[1] Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. Neurology. 2016; 87(20):2123-2131.
[2] Bello L, Gordish-Dressman H, Morgenroth LP, et al. Prednisone/prednisolone and deflazacort regimens in the CINRG Duchenne Natural History Study. *Neurology.* 2015; 85(12):1048-1055.
[3] Gloss D, Moxley RT, Ashwal S, et al. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy: Report of the Guideline Development Subcommittee of the American Academy of Neurology. *Neurology.* 2016; 86:465-472.